Figure 3

Regenerative indications where child with stored cord blood has condition

Disease name	Family count	Family count/total families responding
Autism/apraxia	859	1.99%
Other developmental delay	609	1.41%
Childhood hearing loss	169	0.39%
Cerebral palsy/periventricular leukomalacia/hypotonia	116	0.27%
Diabetes, type 1	114	0.26%
Inflammatory bowel disease	61	0.14%
Hydrocephalus	53	0.12%
Hypoxic-ischemic brain injury	44	0.10%
In-utero brain injury/stroke	38	0.09%
Infant lung disease	30	0.07%
Traumatic brain injury (post-delivery)	24	0.06%
Muscular dystrophy	15	0.03%
Spinal cord injury	15	0.03%
Diabetes, type II	5	0.01%
Systemic lupus	3	0.01%
TOTAL*	**2155**	**4.98%**

*Families may report more than one condition or disease.

("transplant indications") or 15 diseases or conditions primarily under investigation for autologous stem cell infusion ("regenerative indications"), regardless of whether they planned to receive a transplant or infusion.

Results: We received 43,259 completed responses. Of the families completing the questionnaire, 1.87% reported at least one transplant indication and 17.04% reported at least one regenerative indication. Figure 1 shows the total number and percent of families that reported each of the transplant and regenerative indications listed. For transplant indications, after subtracting families where the child with the condition was the family member with stored cells, 1.68% of families reported at least one indication. The most common indications reported were Hodgkin's lymphoma (0.33%), non-Hodgkin's lymphoma (0.32%), and acute lymphoblastic leukemia (0.25%) (Figure 2). Similarly, for regenerative indications, after subtracting families where the child with the condition was not the family member with stored cells, 4.98% of families reported at least one indication. The most common indications reported were autism/apraxia (1.99%), other developmental delay (1.41%), and childhood hearing loss (0.39%) (Figure 3).

Conclusion: Among families who store newborn stem cells in private cord blood banks, conditions for which stem cell transplant or infusion may be indicated or under investigation are relatively prevalent, especially for regenerative indications.

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Unrelated Umbilical Cord Blood Transplant for Diamond-Blackfan Anemia

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Background: Hematopoietic stem cell transplant (HSCT) is the only curative option for patients with Diamond-Blackfan anemia (DBA). However, many patients lack suitable related or unrelated bone marrow donors. Unrelated cord blood transplant (UCBT) has successfully been used to treat many diseases. Advantages of cord blood include: increased likelihood of finding a suitable graft particularly for racial and ethnic minorities, rapid availability, lack of risk to the donor, decreased likelihood of transmitting infections, more permissive HLA mismatching and decreased incidence of graft versus host disease (GVHD). Here we describe a cohort of patients who underwent unrelated umbilical cord blood transplant (UCBT) for DBA at a single center.

Methods: All patients who underwent UCBT for a diagnosis of DBA from 1996 to 2011 at Duke University Medical Center were eligible for this retrospective analysis (n=6). Patients were diagnosed with DBA on the basis of pure red cell aplasia on bone marrow aspirate and when available by ribosomal mutation testing (n=3). Descriptive analysis was used for measures of engraftment, GVHD and survival.

Results: Patients had a median age of 3.1 years (range: 2.5-20.3 years), 3 were male and all were caucasian. Prior to UCBT, patients had a median of 34 (range, 6-230) red cell transfusions and a median ferritin level of 2213ng/ml (range, 319 – 3328 ng/ml). Five of six patients received busulfan (1mg/kg/dose x 16), cyclophosphamide (50mg/kg/dose x 4) and equine Anti-thymocyte globulin (30mg/kg/dose x 3). The most recent patient received fludarabine (30mg/m^2 x 5), melphalan (70mg/m^2 x 2), thiotepa (200mg/m^2 x 1), alemtuzumab (1mg/kg/dose x 3) and hydroxyurea (30mg/kg/day x 13). Patients were HLA matched at 4/6 (n=2), 5/6 (n=2) or 6/6 (n=2) using low resolution HLA-A and –B and high resolution –DRB1 typing. Median total nucleated cell dose pre-cryopreservation was 9.33x10^7 cells/kg (range: 2.3-18.4x10^7 cells/kg). GVHD prophylaxis was cyclosporine and steroids. All patients engrafted neutrophils (median 22 days, range: 19-91 days) and platelets (50K, median 81 days, range: 41-215 days). Two patients experienced grade II-IV acute GVHD and one had extensive chronic GVHD. With a median follow up of 5.5 years (range: 2-14 years), 4 of 6 (67%) patients are alive and well, full donor chimeras, and free of transfusions. One patient died at day 70 from veno-occlusive disease and the second died two years post-transplant from polyserositis, chronic gut GVHD and kidney failure. None of the 4 surviving patients suffer from any major chronic medical problems.

Conclusions: UCBT can successfully be used for the treatment of DBA if otherwise suitable donors are not available. Survival following UCBT is similar to those reported by CIBMTR following related and unrelated adult donor transplants for DBA.[1]

1. Roy, V., et. al. *BBMT*, 2005, 11(8); 600.

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The Contribution of Cardiac Hypertrophy on Transplant Outcome in Patients with HLH Undergoing BMT

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Hemophagocytic Lymphohistiocytosis (HLH) is a rare immunodeficiency disease of early childhood characterized by immune dysregulation due to impaired cytoxic killing and uncontrolled activation of T cells and macrophages. HLH can be congenital or acquired; approximately 40 % of children have an identified genetic defect. The standard approach to HLH is several months of therapy with dexamethasone, cyclosporin and etoposide to achieve disease control, followed by allogeneic hematopoietic stem cell transplant